
iPS cells lead to drug discovery for heart disease, autism up next

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We've long claimed that one ideal role for iPS cells is modeling disease and screening drugs. In fact, we're so committed to that idea we produced a video about it with CIRM grantee Bruce Conklin at the Gladstone Institutes. Scientific American also has a story on disease model their March issue, available online.

Well, a group at Stanford has proven us right. A team led by Ricardo Dolmetsch took skin cells from people with a heart condition called long QT syndrome, reprogrammed those to an embryonic-like state, then matured them into heart muscle cells. These heart cells contracted in the lab dish, but slower and with irregularities compared to similar cells created from people without the heart condition. The work was published online Feb. 9 in *Nature*.

Here's the cool part. The team bathed those cells in a variety of different drugs that have been reported to affect heartbeat rhythms, and found one that restored a regular heartbeat in the diseased cells. The drug, called roscovitine, is currently in clinical trials for a different condition.

According to a Stanford University press release:

“ Dolmetsch cautioned that at this point roscovitine should not be considered an adequate treatment for LQTS - it hasn't been tested for this purpose in living animals, let alone humans, and may have pronounced side effects. Still, he said, it's a promising compound for further drug development. Stanford's Office of Technology Licensing has applied for U.S. patents related to the discovery, and Dolmetsch is starting a new company that intends to license those patents once they're granted.

The primary focus of Dolmetsch's work is autism. The cells he created with irregular heartbeat came from people with a condition called Timothy syndrome, which causes long QT syndrome as well as a form of autism. He has a CIRM Tools and Technologies II award to create iPS cells from people with Timothy syndrome, mature those into neurons and test drugs to find one that improves signs of autism in those cells.

- A.A.

Tags: Stanford University, Heart Disease, Dolmetsch, Autism

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